

### **Non-Technical Abstract.**

Absence of the enzyme purine nucleoside phosphorylase (PNP) in humans causes a condition known as T cell immunodeficiency, an inherited disease which predisposes children to multiple and recurring infections, usually resulting in death of the child by age 10. It is proposed here to test the treatment of PNP deficiency by introduction of a functional PNP gene into lymphocytes, white blood cells responsible for mediating the immunity. Some of the lymphocytes will be removed from the PNP-deficient patient and cultured outside the patients' body. These cells will be exposed to virus (a retrovirus) which has been genetically modified to carry the PNP gene into the lymphocytes, which will then be reinfused into the patient. The patient will then be tested for presence of the PNP gene and enzyme as well as signs of improved immunity. Results from this study will provide an indication of the potential effectiveness of this approach for treatment of PNP deficiency and other related diseases: